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Benitec Biopharma Announces Positive Interim Phase 1b/2a Results for High Dose BB-301 and Continued Durable Improvements for Low Dose BB-301

- Both low dose and high dose BB-301 continue to drive robust improvements in swallowing function for dysphagic Patients with Oculopharyngeal Muscular Dystrophy (OPMD)

- 100% of Patients treated with BB-301 across both doses are formal Responders to therapy

- Interim clinical results for Patients treated with high dose BB-301 demonstrate greater relative improvements at the 3-month post-treatment time-point in throat emptying ability (assessed by videofluoroscopic evaluation of post-swallow food and liquid residue) and functional swallowing capacity (assessed by the Cold-Water Timed Drinking Test)

HAYWARD, Calif., Oct. 28, 2026 (GLOBE NEWSWIRE) -- Benitec Biopharma Inc. (NASDAQ: BNTC) (“Benitec” or “Company”), a clinical-stage, gene therapy-focused, biotechnology company developing novel genetic medicines based on its proprietary “Silence and Replace” DNA-directed RNA interference ("ddRNAi") platform, today announced positive interim clinical results from the ongoing BB-301 Phase 1b/2a first-in-human study (NCT06185673) for Patients treated with high dose BB-301 and continued durable improvements for Patients treated with the low dose of BB-301. The clinical results observed across both dose cohorts of the BB-301 first-in-human study continue to demonstrate robust and durable improvements in swallowing function in dysphagic Patients with OPMD, with a 100% response rate observed across all Patients treated to date.

“Progressive loss of swallowing function has a devastating impact on OPMD Patients, yet there are no approved therapies to address this critical unmet medical need,” said Jerel A. Banks, M.D., Ph.D., Executive Chairman and Chief Executive Officer of Benitec. “The progress we continue to see regarding both the safety and efficacy of BB-301 reinforces our conviction in the program and the potential to establish a new treatment paradigm for Patients suffering from OPMD-related dysphagia. As we advance toward our pivotal study in mid-2027, we remain focused on the promise of delivering a potentially transformative therapy to the OPMD community.”

Key Updated Clinical Study Results

The updated analyses include 12-month post-treatment follow-up results for all six Patients enrolled in Cohort 1 (low dose BB-301) and the available post-treatment follow-up results for all three Patients enrolled in Cohort 2 (high dose BB-301).

Clinical and fluoroscopic results were available for the final 12-month post-treatment follow-

up time-point of the study for all Cohort 1 Patients. Clinical and fluoroscopic results were available for the 9-month post-treatment follow-up time-point for the first Patient enrolled in Cohort 2 and for the 3-month post-treatment follow-up time-point for the second and third Patients enrolled in Cohort 2.

- For Cohort 1, all six Patients are formal Responders to BB-301.
- For Cohort 2, interim clinical results are available, and all three Patients are formal Responders to BB-301.
- Evaluations of dose-related improvements were carried out.
    - Full Cohort comparisons (Cohort 1 vs. Cohort 2) were carried out at the 3-month post-treatment time-point, as complete clinical and fluoroscopic results were available for the total Patient populations of the respective cohorts at this post-treatment time-point.
- A larger proportion of Cohort 2 Patients exhibited improvements in throat emptying ability (assessed by videofluoroscopic evaluation of post-swallow food and liquid residue) and functional swallowing capacity (assessed by the Cold-Water Timed Drinking test) at Month 3 post-BB-301 administration.
    - 67% of Cohort 1 Patients and 100% of Cohort 2 Patients met the statistical criteria for response for improved throat emptying ability (assessed by videofluoroscopic evaluation of post-swallow food and liquid residue) at the 3-month post-treatment time-point.
    - 17% of Cohort 1 Patients and 67% of Cohort 2 Patients met the statistical criteria for response for improved functional swallowing capacity as assessed by Cold-Water Timed Drinking Test (CWDT) at the 3-month post-treatment time-point.
- Improvements in total Sydney Swallow Questionnaire (SSQ) score and throat closing ability for Cohort 1 and Cohort 2 Patients were equivalent at Month 3 post-BB-301 administration.
    - 100% of Patients in Cohort 1 and Cohort 2 met the statistical criteria for response for improved total SSQ score at the 3-month post-treatment time-point.
    - 67% of Patients in Cohort 1 and Cohort 2 met the statistical criteria for response for improved throat closing ability (assessed by videofluoroscopic evaluation) at the 3-month post-treatment time-point.

There have been no Serious Adverse Events in any patient treated with BB-301 to date.

The full poster, accepted as a late-breaking abstract at the Annual Congress of the European Society of Gene and Cell Therapy (ESGCT), is available here.

About OPMD

There are currently no approved therapies for OPMD, a rare autosomal-dominant degenerative muscle disorder, that impacts an estimated 15,000 patients in North America, Europe and Israel. OPMD is caused by a mutation in the poly(A)-binding protein nuclear 1 (PABPN1) gene; PABPN1 is a ubiquitous protein that controls the length of mRNA poly(A) tails, mRNA export from the nucleus and alternative poly(A) site usage. OPMD is a debilitating progressive disease that weakens the pharyngeal muscles, causing severe

swallowing difficulties (dysphagia).[1] Progressive dysphagia impacts 97% of OPMD patients and is a severe, life-threatening complication of OPMD which can lead to chronic choking, malnutrition, aspiration pneumonia and death.

About BB-301

BB-301 is a novel, modified AAV9 capsid expressing a unique, single bifunctional construct promoting co-expression of both codon-optimized Poly-A Binding Protein Nuclear-1 (PABPN1) and two small inhibitory RNAs (siRNAs) against mutant PABPN1 (the causative gene for OPMD). The two siRNAs are modeled into microRNA backbones to silence expression of faulty mutant PABPN1, while allowing expression of the codon-optimized PABPN1 to replace the mutant with a functional version of the protein. BB-301 is administered locally, in a one-time procedure, to the muscles in the throat that drive the swallowing process, an approach designed to maximize local benefit and minimize systemic exposure. We believe the silence and replace mechanism of BB-301 is uniquely positioned for the treatment of OPMD by slowing or halting mutant PABPN1 expression while simultaneously providing a functional replacement protein. BB-301 has received Orphan Drug Designation from the EMA and Orphan Drug and Fast Track Designations from the FDA and is currently being evaluated in a Phase 1b/2a, first-in-human, open-label dose escalation study to evaluate the safety and clinical activity of intramuscular doses of BB-301 administered to subjects with OPMD (NCT06185673).

About Benitec Biopharma Inc.

Benitec Biopharma Inc. (NASDAQ: BNTC) is a clinical-stage biotechnology company developing disease-modifying genetic medicines designed to improve the lives of people with life-threatening, genetically defined diseases. The Company's proprietary "Silence and Replace" DNA-directed RNA interference (ddRNAi) platform combines RNA interference, or RNAi, with gene therapy to create medicines designed to facilitate sustained silencing of disease-causing genes and simultaneous delivery of replacement genes that restore normal cellular function following a single administration. The Company's lead investigational candidate, BB-301, is the first and only disease-modifying genetic medicine in clinical development for the treatment of Oculopharyngeal Muscular Dystrophy (OPMD)-related dysphagia. For additional information, visit www.benitec.com.

Forward Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release include forward-looking statements, within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including statements regarding Benitec's plans to develop and commercialize its product candidates, whether the interim clinical results described in this press release, including the durability of treatment effects observed to date, will be maintained or confirmed with additional follow-up or in later-stage clinical trials, the timing of the completion of preclinical and clinical trials, the anticipated timing of initiation and the design of our planned pivotal study of BB-301, the timing of the availability of data from our clinical trials, the timing and sufficiency of patient enrollment and dosing in clinical trials, the timing of expected regulatory filings and other regulatory steps, the potential for BB-301 to establish a new treatment paradigm for, and to be a transformative therapy for, patients with OPMD-related dysphagia, and the clinical utility and potential attributes and benefits of ddRNAi and Benitec's product candidates, and other forward-looking statements.

Words such as “anticipate”, “believe”, “continue”, “could”, “designed”, “estimate”, “expect”, “intend”, “may”, “plan”, “potential”, “should”, “will” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements are based on the Company’s current expectations and assumptions and are subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: the success of our plans to develop and potentially commercialize our product candidates; the timing of the completion of preclinical studies and clinical trials; the timing and sufficiency of patient enrollment and dosing in ongoing and future clinical trials; the timing of the availability of data from our clinical trials; the timing and outcome of regulatory filings and approvals; the development of novel AAV vectors; our potential future out-licenses and collaborations; the plans of licensees of our technology; the clinical utility and potential attributes and benefits of ddRNAi and our product candidates, including the potential duration of treatment effects and the potential for a “one shot” cure; our intellectual property position and the duration of our patent portfolio; expenses, ongoing losses, future revenue, capital needs and needs for additional financing, and our ability to access additional financing given market conditions and other factors; the length of time over which we expect our cash and cash equivalents to be sufficient to execute on our business plan; unanticipated delays; further research and development and the results of clinical trials possibly being unsuccessful or insufficient to meet applicable regulatory standards or warrant continued development; the ability to enroll sufficient numbers of subjects in clinical trials; determinations made by the FDA and other governmental authorities; regulatory developments in the United States of America; the Company’s ability to protect and enforce its patents and other intellectual property rights; the Company’s dependence on its relationships with its collaboration partners and other third parties; the efficacy or safety of the Company’s products and the products of the Company’s collaboration partners; the acceptance of the Company’s products and the products of the Company’s collaboration partners in the marketplace and market competition; reliance on third party manufacturers and suppliers, and the risks associated with manufacturing complexity, supply interruptions, and compliance with current good manufacturing practices, sales, marketing and distribution requirements for our product candidates; greater than expected expenses; including expenses relating to litigation or strategic activities; our incurrence of significant losses and the uncertainty of our ability to achieve or sustain profitability or generate any revenue; the Company’s ability to satisfy its capital needs through increasing revenue and obtaining additional financing; the impact of local, regional, national and international economic conditions and events; including geopolitical instability, and risks associated with conducting business and seeking regulatory approvals in international markets; our ability to attract and retain key management, scientific, and technical personnel; cyber-security threats and vulnerabilities in our and our third parties' information technology systems; the impact of legislative and regulatory reforms affecting drug pricing and reimbursement, including Medicare drug-price negotiation and government pricing frameworks; potential product liability claims arising from the use of our product candidates in clinical trials or following any future marketing approval; physicians, patients, third-party payers, or others in the medical community may not be receptive to our product candidates, and we may not generate any future revenue from the sale or licensing of our product candidates; disruptions to U.S. government agency operations, including FDA staffing changes; and other risks detailed from time to time in the Company’s reports filed with the Securities and Exchange Commission (the “SEC”), including under the heading

“Risk Factors” in the Company’s Annual Report on Form 10-K for the fiscal year ended June 30, 2026 and subsequent Quarterly Reports on Form 10-Q filed with the SEC. Forward-looking statements speak only as of the date of this press release, and readers are cautioned not to place undue reliance on them. The Company disclaims any intent or obligation to update these forward-looking statements, except as required by law.

References:

1. https://www.mayoclinic.org/diseases-conditions/dysphagia/symptoms-causes/syc-20372028

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